



Substitution form 1449/PTO

INFORMATION DISCLOSURE STATEMENT BY APPLICANT

(Use as many sheets as necessary)

Complete if Known

Application Number	10/586,892
Filing Date	February 9, 2007
First Named Inventor	David S. Lawrence
Art Unit	1636
Examiner Name	Jennifer Ann Dunston
Attorney Docket Number	96700/1160

Sheet 1 of 3

NON PATENT LITERATURE DOCUMENTS

Examiner Initials*	Cite No. ¹	Include name of the author (in CAPITAL LETTERS), title of the article (when appropriate), title of the item (book, magazine, journal, serial, symposium, catalog, etc.), date, page(s), volume-issue number(s), publisher, city and/or country where published.	T ²
	1	BOOMS P et al. "Novel exon skipping mutation in the fibrillin-1 gene: two 'hot spots' for the neonatal Marfan syndrome." Clin. Genet. 55:110-7, 1999. Abstract only	
	2	DU M et al. PTC124 is an orally bioavailable compound that promotes suppression of the human CFTR-G542X nonsense allele in a CF mouse model. PNAS 105: 2064-69, February 2008.	
	3	DURIEZ B et al. "An exon-skipping mutation in the btk gene of a patient with X-linked agammaglobulinemia and isolated growth hormone deficiency." FEBS Lett. 1994 June 13;346(2-3):165-70. Abstract only	
	4	ENZMANN H et al. Damage to mitochondrial DNA induced by the quinolone Bay y 3118 in embryonic turkey liver. Mutat. Res. 425:213-24, 1999. Abstract only	
	5	FERNANDES R et al. Incorporation of structurally defective type II collagen into cartilage matrix in kniest chondrodysplasia. Arch Biochem Biophys 355:282-90 1998 Abstract	
	6	FOGLI A et al. "Intracellular levels of the LIS1 protein correlate with clinical and neurobiological findings in patients with classical lissencephaly." Ann Neurol. 1999 Feb;45(2):154-61. Abstract only	
	7	GUILFORD P et al. "E-cadherin germline mutations in familial gastric cancer." Nature 1998 Mar. 26;392(6674):402-5. Abstract only	
	8	HIRANO M et al. Dominant negative effect of GTP cyclohydrolase I mutations in dopa-responsive hereditary progressive dystonia. Ann Neurol. 44:365-71 1998 Abstract	

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1 Applicant's unique citation designation number (optional). 2 Applicant is to place a check mark here if English language Translation is attached.

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	9	HIRAWAT S et al. Safety, tolerability, and pharmacokinetics of PTC124, a nonaminoglycoside nonsense mutation suppressor, following single- and multiple-dose administration to healthy male and female adult volunteers. Clin. Pharm. 2007; 47:430-444.	
	10	KEREM E et al. Effectiveness of PTC124 treatment of cystic fibrosis caused by nonsense mutations: a prospective phase II trial. The Lancet vol. 372: 719-27, 2008.	
	11	LAUMONNIER Fet al. X-Linked mental retardation and autism are associated with a mutation in the NLGN4 gene, a member of the neuroligin family. Am J Hum. Genet. 74:552-7, 2004.	
	12	MACOSKA J et al. "Loss of expression of human spectrin Src homology domain binding protein 1 is associated with 10p loss in human prostatic adenocarcinoma."	
		Neoplasia, 3:99-104, 2001.	
	13	MERCURI E et al. "Muscle MRI findings in a three-generation family affected by Bethlem myopathy." Eur. J. Paediatr. Neurol. 2002;6(6):309-14. Abstract only	
	14	NICHOLLS A et al. "An exon skipping mutation of a type V collagen gene (COL5A1) in Ehlers-Danlos syndrome." J. Med. Genet. 1996;33:940-946.	
	15	THOMAS A et al. "Electrochemical characteristics of five quinolone drugs and their effect on DNA damage and repair in Escherichia coli." J. Antimicrob. Chemother. 1990	
		May;25(5):733-44. Abstract only	

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	16	Tiller G et al. "A recurrent RNA-splicing mutation in the SEDL gene causes X-linked spondyloepiphyseal dysplasia tarda." Am. J. Hum. Genet. 68:1398-1407, 2001.	
	17	SPAYDE E et al. "Exon skipping mutation in the COL9A2 gene in a family with multiple epiphyseal dysplasia." Matrix Biol. 19:121-8 2000. Abstract only	
	18	VAILLY et al. "Identification of a homozygous exon-skipping mutation in the LAMC2 gene in a patient with Herlitz's junctional epidermolysis bullosa."	
		J. Invest. Dermatol. 1995 Mar;104(3):434-7. Abstract only	
	19	WELCH E et al. "PTC124 targets genetic disorders caused by nonsense mutations." Nature 447:87-91, 2007.	
	20	YEOWELL H et al. "Ehlers-Danlos syndrome type VI results from a nonsense mutation and a splice site-mediated exon-skipping mutation in the lysyl hydroxylase gene."	
		Proc. Assoc. Am. Physicians 1997 Jul;109(4):383-96. Abstract only	
	21	Selleck Chemicals, online catalog, PTC124 (Ataluren) 2010	

Examiner Signature	/Jennifer Dunston/ (06/17/2010)	Date Considered	
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